STRATEGIES FOR MEDICAL TECHNOLOGY ASSESSMENT

SEPTEMBER 1982

Summary

CONGRESS OF THE UNITED STATES
Office of Technology Assessment
Washington, D. C. 20510

J952-38

Office of Technology Assessment

Congressional Board of the 97th Congress

TED STEVENS, *Alaska, Chairman*

MORRIS K. UDALL, *Arizona, Vice Chairman*

Senate

ORRIN G. HATCH
Utah

CHARLES McC. MATHIAS, JR.
Maryland

EDWARD M. KENNEDY
Massachusetts

ERNEST F. HOLLINGS
South Carolina

HOWARD W. CANNON
Nevada

House

GEORGE E. BROWN, JR.
California

JOHN D. DINGELL
Michigan

LARRY WINN, JR.
Kansas

CLARENCE E. MILLER
Ohio

COOPER EVANS
Iowa

JOHN H. GIBBONS
(Nonvoting)

Advisory Council

CHARLES N. KIMBALL, *Chairman*
Midwest Research Institute

EARL BEISTLINE
University of Alaska

CHARLES A. BOWSHER
General Accounting Office

CLAIRE T. DEDRICK
California Land Commission

JAMES C. FLETCHER
University of Pittsburgh

S. DAVID FREEMAN
Tennessee Valley Authority

GILBERT GUDE
Library of Congress

CARL N. HODGES
University of Arizona

RACHEL McCULLOCH
University of Wisconsin

WILLIAM J. PERRY
Hambrecht & Quist

DAVID S. POTTER
General Motors Corp.

LEWIS THOMAS
Memorial Sloan-Kettering Cancer Center

Director

JOHN H. GIBBONS

The Technology Assessment Board approves the release of this report. The views expressed in this report are not necessarily those of the Board, OTA Advisory Council, or of individual members thereof.

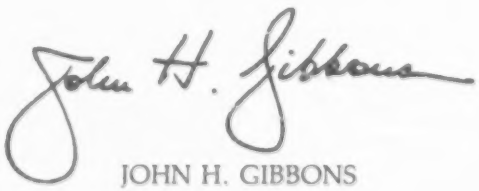
Foreword

This summary presents the findings of OTA's study *Strategies for Medical Technology Assessment*. OTA began the study in July 1980, at the request of the House Committee on Energy and Commerce.

The full report focuses on the flow of information that is central to an effective assessment system. It critiques the current assessment system with respect to identifying medical technologies for assessment; developing and synthesizing information about such technologies' health, economic, and social effects; and disseminating this information to health care decisionmakers. This summary presents the highlights of that critique. It also provides a list of policy options, both legislative and oversight, for Congress to improve the system.

During the course of this assessment, both the House Committee on Energy and Commerce and the Senate Committee on Labor and Human Resources requested that OTA study several specific areas in more depth. In response to these requests, OTA is publishing three other volumes: 1) a report on medical technology under proposals to increase competition in health care, 2) a report on the postmarketing surveillance of prescription drugs, and 3) a technical memorandum on MEDLARS (the National Library of Medicine's Medical Literature Analysis and Retrieval System) and health information policy. Another paper, funded as part of this assessment, concerns the potential role of Professional Standards Review Organizations in medical technology assessment.

Drafts of the final report were reviewed by the advisory panel chaired by Dr. Lester Breslow, by the Health Program Advisory Committee chaired by Dr. Sidney S. Lee, and by approximately 100 other individuals and groups representing a wide range of disciplines and perspectives. We are grateful for their many contributions. As with all OTA reports, however, the content is the responsibility of the Office and does not constitute consensus or endorsement by the advisory panel or the Technology Assessment Board.



JOHN H. GIBBONS
Director

98-143 O - 82 - 2

Advisory Panel for Strategies for Medical Technology Assessment

Lester Breslow, *Panel Chairman*
School of Public Health, University of California, Los Angeles

Morris Collen
Director of Technology Assessment
Kaiser Permanente Medical Group

Richard Cooper
Williams & Connolly, Inc.

D. V. d'Arbeloff
Chairman and Chief Executive Officer
Millipore Corp.

Harvey Fineberg
Harvard School of Public Health

Jerome D. Frank
The Henry Phipps Psychiatric Clinic
The Johns Hopkins Hospital

William Goffman
School of Library Science
Case Western Reserve University

Leon Greene
Vice President
New Product Technology
Smith, Kline & French Laboratories, Inc.

David B. Horner
Orthopedic Surgeon

Stanley B. Jones
Vice President
Blue Cross/Blue Shield Association

F. Wilfrid Lancaster
Graduate School of Library and Information Science
University of Illinois

Louise B. Russell
Senior Fellow
The Brookings Institution

Herbert Semmel
President
Consumer Coalition for Health

Robert M. Veatch
Kennedy Institute of Ethics
Georgetown University

Richard W. Vilter
American College of Physicians
College of Medicine
University of Cincinnati

Kenneth E. Warner
School of Public Health
University of Michigan

Richard N. Watkins
Staff Physician
Group Health Cooperative

Carol Weiss
Graduate School of Education
Harvard University

Kerr L. White
Deputy Director for Health Sciences
Rockefeller Foundation

OTA Project Staff—Strategies for Medical Technology Assessment

H. David Banta, *Assistant Director, OTA Health and Life Sciences Division*

Clyde J. Behney, *Health Program Manager*

Bryan R. Luce, *Project Director*
Dale A. Carlson, *Analyst*
John C. Langenbrunner, *Analyst*
Gloria Ruby, *Analyst*
Kerry Britten Kemp, *Editor*

Virginia Cwalina, *Administrative Assistant*
Mary E. Harvey, *Secretary*
Pamela Simerly, *Secretary*
Lorraine G. Ferris, *Secretary**
Nancy L. Kenney, *Secretary***

Other Contributing Staff

Ann Rose, *Senior Analyst*
Arthur Kohrman, *Congressional Fellow*
Hellen Gelband, *Analyst*
Mark Hochler, *Intern*
Lisa Scheffler, *Intern*
Harvey Sepler, *Intern*

Principal Contractors

Rand Corp.
John P. Bunker, *Stanford University*
Lawrence Miike
John B. Reiss, *Baker and Hostetler*
Leonard Saxe, *Boston University*
Paul M. Wortman, *University of Michigan*

OTA Publishing Staff

John C. Holmes, *Publishing Officer*

John Bergling Kathie S. Boss Debra M. Datcher Joe Henson

*Until January 1982.
**Until September 1981.

Strategies for Medical Technology Assessment

Knowledge advances by steps, and not by leaps.

—*Thomas Babbington Macaulay*

NEED FOR A STRATEGY

Several reasons for assessing medical technologies have been presented in previous OTA reports, *Assessing the Efficacy and Safety of Medical Technologies* and *The Implications of Cost-Effectiveness Analysis of Medical Technology*. The main reasons are to help ensure that medical technologies are safe, efficacious, and appropriately used. Whether current policies and practices for medical technology assessment achieve these and related objectives is the subject of this report. Having studied both the methods of medical technology assessment and the dissemination of information developed by technology assessment, OTA finds that a strategy is needed to implement the assessment process to make it more effective. OTA also finds that greater attention to assessment of social and ethical values is needed for policymaking.

A *medical technology,* as used in this report, is a drug, device, or medical or surgical procedure used in medical care. (The term may also apply to the organizational and supportive systems within which medical care is delivered, but those systems are not the focus of this report.) *Medical technology assessment* is, in a narrow sense, the evaluation or testing of a technology for safety and efficacy. In a broader sense, it is a process of policy research that examines the short- and long-term consequences of individual medical technologies and thereby becomes the source of information needed by policymakers in formulating regulations and legislation, by industry in developing products, by health professionals in treating and serving patients, and by consumers in making personal health decisions. Unfortunately, that process currently has deficiencies that cause or allow confusion to exist at all decision points.

Historically, medical technology assessment has developed incrementally as responses to specific demands. Taken singly, some of these responses have been coherent (e.g., the Food and Drug Administration's (FDA's) premarketing approval process which was developed to protect the public from unsafe and inefficacious new drugs). Taken in combination, however, these various responses do not constitute a coherent system for assessing all classes of medical technologies. The present approach is characterized by multiple participants from the public and private sectors, and by uncoordinated activities. Complicating matters further is the large number of medical tech-

nologies in use, with thousands of new technologies appearing every year. The result is an overload and confusion among decisionmakers and consumers.

OTA finds that a strategy is needed to guide the selection and implementation of components that would constitute a coordinated system of medical technology assessment. The basis of the strategy should be the values and available resources in a free-market economy, coupled with the social responsibility to make available safe, effective medical care. The vehicle of the strategy should be a systematic process of information development, dissemination, and use. The target should be to address the confusion deriving from the lack of information available to decisionmakers.

Minimally, the following components of an assessment system must be considered in developing a strategy:

1. the values of individuals and of society concerning medical technologies and their use;
2. the goals and appropriate role of medical technology assessment in society;
3. the types of assessment information needed for decisionmaking;
4. the methods and technologies for developing and acquiring the information; and
5. mechanisms for disseminating and applying the information, including programs that will use the information.

A strategy for assessing medical technologies must consider not only the methods of assessment, but also the needs, demands, and resistances of potential participants in the process of assessment. Specifically, the public itself as consumers; health care professionals as users; industry as innovators, producers, and reimbursers; and the Federal Government simultaneously as purchaser and guardian must be informed and active in setting mutually compatible goals for technology assessment. Each sector has health, social, and economic values underlying its decisionmaking. Clarifying those values and realistically accommodating them will require developing not just more, but also more reliable, information about the safety, efficacy, cost effectiveness, and social and ethical implications of all classes of medical technologies. The inconsistencies and contradictions in available information are reflected in the inconsistent and competing pressures from the various sectors.

DIMENSIONS OF THE NEED AND THE PROBLEM

Most medical technologies have a therapeutic or diagnostic value for specific problems under appropriate circumstances. The difficulty is determining for whom and under what circumstances use of a technology is valid or worth the tradeoff of risks and benefits. Mammography and radical mastectomy, for example, have a place in the detection and treatment of breast cancer, but understanding exactly what that place is may take years and a certain amount of trial and error.

Government

The Federal Government's interest in developing clear policies and an effective strategy for assessing medical technology derives from its traditional role as guardian of the public's safety and of social equity and from its concerns about economic issues. As protector of the public, the Government seeks to ensure that health care is not only safe but also efficacious. As the single largest buyer of health services, the Government seeks to ensure that all citizens, especially the poor, have health care available to them; but the Government is also concerned about rising health care costs in general and specifically about those it pays for directly through programs of service or reimbursement (Medicare, for example) and through biomedical and other health research. Any policies the Government sets will affect not only the Government itself, but the public and private industry, and such policies must especially be justifiable when the public and private industry make self-interested demands.

The Public

The public is often neither fully informed about the safety and efficacy of individual technologies nor educated about the issues of cost and social values that must be considered in the adoption of a technology. The public mixes facts with beliefs, hopes, and fears and translates those into confused, contradictory, and often impossible demands.

For example, the public hears of a drug, perhaps one used in another country, and wants it immediately available to patients in the United States, especially when available therapies are ineffective. The desperation individuals feel tends to outweigh the fear of any risks that might be involved, and they demand the right to take personal responsibility for use of the drug. Perhaps assuming that if a therapy is used in a European country, it has already met rigorous assessment standards, the public perceives itself as being denied a cure for no valid reason.

Simultaneously to demanding speedy availability and personal responsibility, however, the public demands protection against all forms of unsafe medical practice and is prepared to sue for mistakes. Perhaps because of the rigor which FDA applies to approval of new drugs and because of Government safety standards applied to so many nonmedical products, the public assumes that it is likewise protected in undergoing any medical or surgical procedure recommended.

The confused demands of the public can be viewed either as irrational or as a frustrated reflection of the deficiencies that do exist in the Nation's approach to assuring the availability of safe, effective, and cost-effective medical technologies. Numerous needs and values are implied in the demands of the public and must be taken into account when planning a strategy of policies and procedures for medical technology assessment.

Health Professionals

Health professionals often find themselves in circumstances that require decisions based on inadequate information. The flaws in the information flow to physicians and other health professionals are numerous: there is not enough information available about the safety, efficacy, costs, and social values of medical technologies; much existing information is of dubious quality and is therefore unreliable; the practical significance of data is usually not interpreted for clinicians; and easy access to the appropriate information is rare.

Furthermore, medical education typically does not train physicians and other health care professionals to make decisions based on a consideration of values. They are trained to seek the most reliable technique to produce a desired physiological response. As an illustration, in the issue of saving the lives of extremely premature babies in incubators, physicians, by training, would tend to be concerned mainly with choosing the technology that would support life. Physicians would less likely know or be concerned about the implications of the survival of the deformed or retarded infant—implications for the infant itself, for the family, and for society. Thus, developing and supplying the right kind of assessment information to health care professionals is essential to a strategy for medical technology assessment.

Industry

From the point of view of the private sector, of producers of technologies, and of third-party payers, the assessment of medical technologies is both advantageous and disadvantageous. Government's involvement in the assessment process raises primarily financial issues for the private sector.

Industry, which invests money in research and development (R&D), is willing to do so if there is a potential market for the device or drug; however, excessive regulation or the wrong kind of regulation by the Government could discourage innovation if companies fear that assessment will ultimately preclude marketing their product or making a profit from it.

Private third-party payers, on the other hand, might welcome shifting the entire burden of assessment to the Federal Government. They must make decisions about reimbursement—whether to reimburse for specific procedures and if so how much—but they have little incentive to conduct their own assessments of procedures because of the expense. Assessment information tends to be widely available and not proprietary; the insurance companies cannot profit individually from conducting assessments. The failure of industry members to adequately conduct assessment activities on their own puts a heavy responsibility in the Government domain.

Nature of the Challenge

The market for medical technologies is moderated by individual consumer tastes and financial constraints. To perhaps a greater degree, it is influenced

by policies that determine what kinds of research will be supported, what regulations restrict market entry, and which technologies will be reimbursable by Government or private programs.

No policy decision has isolated effects in just one sector; repercussions occur throughout the entire social and economic fabric of the Nation. A regulatory decision to require extensive, expensive assessment of a medical device in a developmental phase, but not to offer industry assistance in the assessment, for example, could lead to a decision by industry never to begin the innovation phase. An idea might never be realized which eventually could have best served the public. In fact, current policies and procedures for assessment are not adequate to fully serve the public interest. No consistent pc icy or system exists for assessing all classes of medical technologies, nor even for various technologies within a class.

Reimbursement decisions also influence the innovation and adoption of medical and surgical procedures. Although new procedures tend to be adopted and reimbursed without adequate assessment, in the case of truly innovative procedures, third-party payers sometimes refuse reimbursement. While encouraging new applications, slight modifications, and excessive use of existing technologies, the present reimbursement system may discourage radical innovations.

The challenge in developing a strategy for assessment is to develop a system that will serve the public interest by encouraging the development and appropriate use of needed and safe medical technologies without unnecessarily discouraging innovation and production.

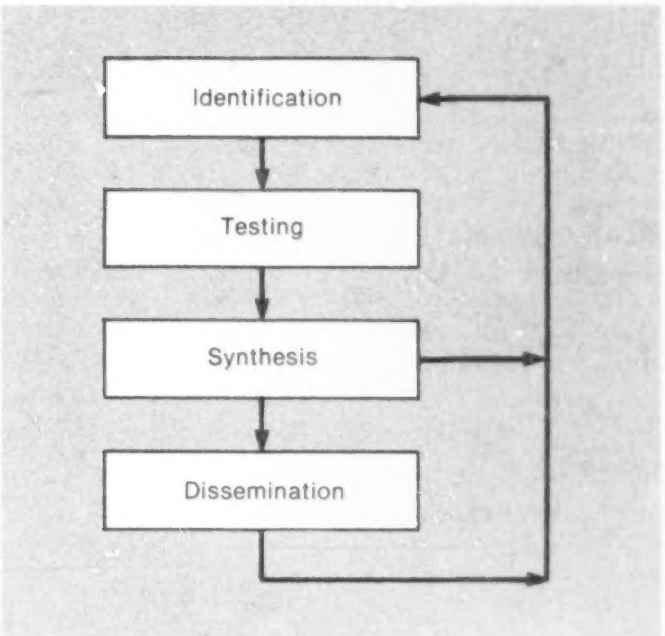
CONCEPTUAL FRAMEWORK FOR MEDICAL TECHNOLOGY ASSESSMENT

Medical technology assessment involves numerous components and subcomponents at various stages of the process. Though these do not exist as a coherent system, discussion of them is facilitated by describing a systematic framework. The multiple components of the medical technology assessment process can be conceptualized as an information flow associated with the following four stages of assessment (see fig. 1):

- *Identification.*—Monitoring technologies, determining which need to be studied, and deciding which to study.
- *Testing.*—Conducting the appropriate analyses or trials.
- *Synthesis.*—Collecting and interpreting existing information and the results of the testing stage, and, usually, making recommendations or judgments about appropriate use.
- *Dissemination.*—Providing the synthesis of information, or any other relevant information, to the appropriate parties who use medical technologies or make decisions about their use.

This four-stage process is applicable to the three classes of medical technologies mentioned earlier—namely, drugs, devices, and medical and surgi-

Figure 1.—Process of Assessing Medical Technologies



SOURCE: Office of Technology Assessment.

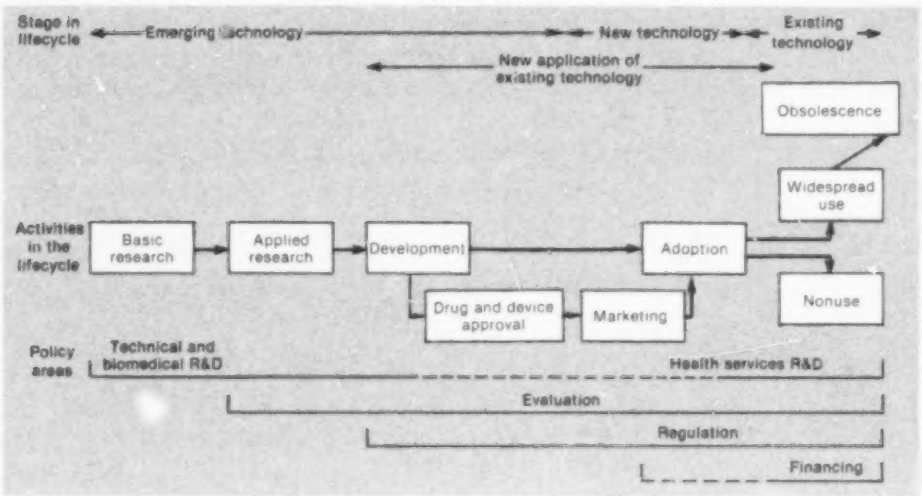
cal procedures. It is also applicable to any technology in any of four typical stages of development, loosely defined as follows:

- *Emerging technology.*—A technology in the phase prior to adoption.
- *New technology.*—A technology in the phase of adoption.
- *Existing technology.*—A technology in general use.
- *New application of an existing technology.*—A new application of a technology in general use.

Visualizing the lifecycle of a hypothetical technology (see fig. 2) makes obvious some of the decision points at which assessment information is essential. If an emerging technology is a drug or device, industry must decide whether to commit resources to develop it; must later decide whether to market it; and must ultimately decide whether to maintain, alter, or discard it. If a new drug or a certain class of device is to be marketed, FDA must decide whether to grant market approval based on safety and efficacy criteria. If the new technology is to be used in medical practice, someone must decide whether to pay for it. In some cases, the Health Care Financing Administration (HCFA) must decide whether to include a new technology or a new use of an existing technology as a reimbursable expense for Medicare beneficiaries. Private insurers, such as Blue Cross/Blue Shield and health maintenance organizations, must make similar decisions. Hospitals must decide whether to purchase, and practitioners and their patients must decide whether to use, the technology.

98-143 O - 82 - 3

Figure 2.—Lifecycle of a Medical Technology



SOURCE: Office of Technology Assessment.

In contrast to drugs and devices, medical and surgical procedures and their variations are ordinarily developed by clinicians and researchers and therefore seldom require investment decisionmaking by industry. Furthermore, under the present system, medical and surgical procedures are not regulated for safety and efficacy by FDA and thus tend to escape the regulatory decisions. Nevertheless, decisions about the use of such procedures must be made.

Many medical technologies in use have not been adequately evaluated. If all medical technologies were adequately assessed as emerging or new technologies, there would be less need for assessing existing technologies.

In addition to considering the stages of the assessment process and the classes and developmental stages of technologies, an assessment system requires the measuring of specified effects. Depending on the technology, the effects to be considered are health (safety, efficacy, and effectiveness), economic, or social. Once the categories of effects to measure have been determined, testing and analysis may begin. Throughout the assessment process, all information and decisions must be balanced against the moral and ethical values of society.

IDENTIFICATION: TECHNOLOGIES NEEDING ASSESSMENT

A decision to conduct a technology assessment must be preceded by the identification of technologies that should be assessed and the setting of priorities among candidate technologies. Identification procedures may vary with the type of technology, but basically can be classified as one of three types: 1) routine mechanisms, 2) priority-setting mechanisms, and 3) mech-

anisms of opportunity. Routine mechanisms systematically identify a class of technologies, usually in relation to a specific event—e.g., FDA requires that all drugs and devices be registered before they can be marketed or tested in humans. Priority-setting mechanisms are used, as needed, to apply implicit or explicit criteria to determine which technologies should be assessed—e.g., HCFA and the National Institutes of Health (NIH) set research agendas. Mechanisms of opportunity are not formalized but are valuable in identifying technologies as they surface or become important—e.g., patient outcome data may bring the need for analysis to the attention of researchers or the public.

Identifying medical technologies for priority-setting and assessment is an important responsibility primarily of several agencies within the Department of Health and Human Services (DHHS): FDA, the National Center for Health Services Research (NCHSR), NIH, and HCFA. The National Center for Health Care Technology (NCHCT), while it was funded, also identified technologies for assessment.

FDA identifies new drugs and medical devices through its premarket approval authority. To test promising new drugs in humans, drug sponsors (e.g., manufacturers) must notify and receive permission from FDA through a "notice of claimed investigational exemption for a new drug" (IND). If the drug successfully passes this premarket testing, the sponsor may file for a "new drug application" (NDA), which is a request for FDA's permission to market the drug. Since 1962, when this regulatory mechanism was instituted, FDA has reviewed over 13,500 applications for INDs and has approved about 1,000 NDAs. In 1976, FDA was also given expanded responsibility for regulating medical devices. In the first 4 years of implementing the 1976 Medical Device Amendments, about 98 percent of the listed devices in the 10,540 premarket notifications received were claimed to be "substantially equivalent" to preexisting devices. In 1981, FDA estimated that 2,300 premarket notifications would be reviewed. New applications of existing drugs and devices must also meet premarket approval requirements, but the initiative for these new applications remains with the manufacturer, not with FDA. FDA does support some monitoring activities of existing drugs and requires manufacturers to report adverse reactions, but these postmarketing surveillance activities are focused on the safety aspects of these drugs, not on refinements in use or new uses.* Nevertheless, postmarketing surveillance has the potential of becoming an effective method of identifying existing technologies in need of further assessment.

NIH and *NCHSR* are research agencies that identify emerging and sometimes new technologies in need of assessment through their priority-setting processes for research grants and contracts. Projects are selected on the basis of technical merit and whether they are addressing important issues. The processes generate information useful to policy decisions, but do not necessarily address the immediate priorities of operating agencies such as HCFA.

HCFA reimburses for Medicare and therefore has obvious incentives for identifying technologies in need of assessment; nevertheless, it has no mech-

*This topic is to be explored in greater depth in OTA's forthcoming report, to be published this fall, entitled *Postmarketing Surveillance of Prescription Drugs*.

anisms for the identification of existing technologies in widespread use. For new technologies, the identification is by opportunity. When the question of coverage arises for new technologies, HCFA must determine whether it has adequate information to make a decision and must set priorities for technologies that must be assessed to provide more information. Also, through its Office of Research and Demonstrations, HCFA sets priorities for assessing technologies that are important to its operations.

NCHCT was established in 1978 to undertake and support assessments of medical technology, but did not receive funding in 1982. NCHCT coordinated interagency issues, but also set its priorities internally and had its own responsibilities for identifying technologies. Specifically, NCHCT compiled an annual "emerging technology list" as an early alert system for assessment, but the 1981 reauthorization of NCHCT withdrew its authority to compile the list. (Industry argued that the list threatened innovation by casting doubt on the eventual marketability of a technology.) NCHCT also initiated a plan to develop a joint public-private model for collecting clinical data on emerging technologies. Finally, the NCHCT Director chaired the Technology Coordinating Committee of DHHS, which was the Department's primary mechanism for coordination of issues associated with medical technologies.

Overall, the identification stage of the current system of technology assessment has serious shortcomings. The degree to which current processes identify technologies varies. Emerging and new drugs and devices are adequately identified for assessment prior to their being marketed. However, emerging and new medical and surgical procedures are not adequately identified, because no one in either the private or the Government sector has a clear responsibility for the task. New mechanisms are especially needed to identify for the purpose of assessment existing technologies of all classes, new applications of existing technologies of all classes, and medical and surgical procedures in all four stages of development.

TESTING: TYPES OF INFORMATION NEEDED AND MECHANISMS FOR TESTING

As a basis for decisions, a strategy to assess medical technologies must take into account what is known, what is not known, what is needed, what can be obtained, and at what cost. Information will never be perfect, and money and time will always be limited; thus, evaluation methods must be used judiciously and their results must be interpreted cautiously, in conjunction with numerous other measurements, especially with consideration for society's moral and ethical values. Three categories of information about a medical technology are needed for policy decisions: 1) health effects, 2) economic effects, and 3) social effects. The methods and procedures for determining these effects have strengths and weaknesses closely paralleling those of the identification phase.

Health Effects

Health effects are determined during the testing stage of assessment. The basic questions asked are: Does the technology work? and How well does it work? The former question seeks information about the technology's efficacy and effectiveness, the latter about its safety (and risk). The information provided by analyses of health effects helps decisionmakers determine whether a drug or device should be allowed on the market or whether further investment in R&D is warranted.

Patient outcome is the desired endpoint measured in efficacy and effectiveness analyses; efficacy is tested under ideal clinical conditions, whereas effectiveness is tested under average, or typical, conditions. Tests for effectiveness demonstrate whether efficacy information can be generalized to the population at large. For new drugs and certain devices in the emerging phase, efficacy must be established in preclinical, biochemical, or animal tests before it can be tested among humans. The method that gives the most valid and most reliable information about efficacy is the randomized clinical trial (RCT). The strength of the RCT lies in its randomization process, producing two or more groups that are identical except for chance occurrence, which can be estimated statistically. The drawbacks of RCTs are that they can only be used in certain settings, they are sometimes not ethical to conduct, and they do not always provide complete information about safety.

Thus, despite the highly valid information they can produce, RCTs are not always the method of choice. Other methods can be used as substitutes for RCTs or to supplement them. Observational methods, for example, are designed to analyze data from nonrandomized study designs. Such methods can be useful in ruling out competing explanations for an observed effect and for testing hypotheses in large, diverse populations after a technology is widely diffused. Prospective cohort studies, for example, can be used to detect rare adverse reactions to drugs that were unsuspected prior to marketing. Case-control studies are an inexpensive means of indicating whether the use of a technology results in a small level of risk.

Another, more common type of study is the case study, typified by a physician reporting his or her experience with particular technologies and patients. Case studies are useful in an overall assessment strategy in that they can facilitate the identification of technologies in need of assessment. Case studies are important identification mechanisms of opportunity, as defined earlier. However, the validity of case studies is extremely low because of, among other things, observer bias and the placebo effect. Nevertheless, clinicians are very often swayed by these case reports, which fill the medical literature and which often describe the successful application of a technology.

Safety is measured in terms of a risk-to-benefit ratio; it is therefore a relative concept. The estimation of safety may be a byproduct of testing for efficacy and effectiveness. A low risk may be unacceptable if there is no benefit, but a high risk may be acceptable if the benefits are also high. RCTs

tend to give risk information only on a small segment of the population. To generalize to other segments, supplemental information is needed from surveys and methods which can make use of registries and clinical data banks.

For certain technologies, especially devices, establishing the technology's performance integrity is a prerequisite for efficacy assessment. Performance standards usually pertain to the chemical, physical, and electric properties of devices. Similar standards are often used in evaluating technologies which have an intermediate rather than a direct effect on the patient's health outcome, e.g., diagnostic and often prevention technologies. In such cases, the technology is evaluated in terms of its ability to cause one effect that in turn will cause the desired result. For example, an automatic blood pressure monitoring device must accurately measure and record blood pressure if it is to be used for diagnostic purposes.

No precise formula exists for choosing the best or most appropriate evaluation method. The stage of development of the technology itself—e.g., emerging, new, or existing—will partially determine the appropriateness of a method. The purpose of the technology—e.g., diagnostic, therapeutic, or preventive—will limit the range of appropriate methods. However, other factors such as existing knowledge about the risks and benefits and available resources may influence or override otherwise "ideal" choices. The important criterion in selecting analytic methods is not which is theoretically more sophisticated, but which is practically the most appropriate.

Economic Effects

Analytical methods to determine the economic effects of medical technologies comprise a spectrum ranging from sophisticated computer-based data analyses to best-guess estimates of costs and benefits. The broad terms cost-benefit analysis (CBA) and cost-effectiveness analysis (CEA) refer to two techniques for comparing the positive and negative consequences of alternative ways to allocate resources. The principal distinction between the two is that CBA values all costs and benefits in monetary terms whereas CEA produces a measure of the cost involved in terms of some desirable health-related effects (e.g., years of life gained).

Measurements of economic effects should consider both direct and indirect costs. Direct costs are those associated with direct medical care usage: the cost of the physician, the hospital, the medical supplies. Indirect costs are associated with the value of time lost in receiving medical care and in being sick. When indirect costs are considered in economic analyses—and often they are not—they are frequently measured in terms of lost or gained wages.

Economic analysis is complex and must consider more than charges for services. For example, cost analyses should develop information on opportunity cost, marginal valuation, joint production considerations, R&D costs, overhead, costs v. prices, and discounting. Just as no one method is invariably appropriate in the evaluation of health effects, no one method of economic analysis is appropriate. The user of the information will partial-

ly determine the kinds of analyses done. For the patient, the actual cost of services is the important information. For policymakers, more complex information is required. In the sequence of the assessment process, information about the economic effects may be useless if reliable and appropriate information about the health effects is not available.

Social Effects

Urgent ethical and social questions are being raised in areas of biomedicine such as experimentation with human subjects, genetic engineering, human reproduction, and the possibly inappropriate prolongation of life. Who is affected by a medical technology? Who is not affected? What values of individuals and society are involved in use of the technology? What ethical principles are involved in testing the technology?

To varying degrees, medical technologies may affect the personal and work lives of patients and their families; influence the structure of medical, legal, and economic systems; and challenge society's most fundamental beliefs. Considerations of the social and ethical implications of medical technologies, therefore, must take an important place in the development of policies. Social implications are the direct or indirect effects of medical technology on the concepts, relationships, and institutions society considers important. Ethical questions in relation to medical technologies—especially those concerning principles of distributive justice, respect for individuals, and benevolence—may also have profound social implications.

Unlike health and economic effects, social and ethical issues do not lend themselves to quantitative measurement and analysis. However, the systematic identification and evaluation of the social impacts resulting from the use of medical technologies can be crucial. A related task is to identify the values that underlie policy alternatives, including moral and ethical values. Systematically assessing values does not necessarily elucidate a single, clear, conclusive answer about which policy to adopt; but, rather, it clarifies the array of choices, the reasons for disagreements, and the compromises required.

A second aspect of assessing values is to make a reasonable inquiry into the values that permeate and underlie the assessment itself. Value judgments enter into every aspect of technology assessment; they determine which technologies will be assessed and at what phase of their development, the scope of assessments, the kinds of data that will be collected and analyzed, the methods of the assessment, and how the assessment findings will be used in decisionmaking. It is important to clarify, therefore, why an assessment of a particular technology was initiated and how it fits into larger cultural and political contexts, what affects the performance of assessment (e.g., the choice of assessors and the analytic goals and methods), and what values affect the application of the results.

Mechanisms for Testing

The major problem with the testing phase of the current assessment system is the lack of a systematic approach for testing identified technologies in all phases of development for all types of required information.

FDA, in its regulatory role, is probably the most significant agency in stimulating technology testing. Most FDA regulation requires industry to test, according to approved protocols, new drugs and many medical devices for safety and efficacy. For drugs, Phase I studies are studies to determine levels of tolerance (toxicity), followed by early dose ranging studies for safety and sometimes efficacy. If safe, the drug can be tested in Phase II studies to demonstrate efficacy and relative safety under controlled conditions. Phase III studies are expanded controlled and uncontrolled clinical trials. If these trials are successful, the company may file an NDA. FDA then reviews the data and may approve the drug for marketing. Since 1962, FDA has approved about 1,000 NDAs. For devices, FDA requires that 90 days notice be given about any new device industry intends to market. If a device does not meet safety and performance standards for its assigned classification, or if adequate information is not available for such a determination, FDA may require testing of the device. For drugs and devices, FDA's assessment activities are generally limited to safety and efficacy and do not involve cost, cost effectiveness, or social effects.

Unlike drugs and devices, medical and surgical procedures are not regulated, and their testing, if done, is through research whose funding comes primarily from NIH and from private foundations. The costs of the later developmental phase of procedures tend to be paid by patients (or by the Government), usually through standard medical insurance policies, even when the procedure has been clearly designated as experimental. Medical and surgical procedures usually begin as user-generated innovations; for example, a surgeon may modify an existing technique during surgery. Increasingly, innovations arise in academic centers, from researchers who know how to present their innovations in a technically acceptable manner at professional meetings and in journals. These researchers' presentations tend to encourage the adoption of innovations without their receiving a routine, formal examination for safety and efficacy.

Whereas FDA regulations affect efficacy and safety, four other regulatory programs are concerned with cost issues: section 1122 review, State certificate-of-need laws, the National Health Planning and Resources Development Act of 1974, and Professional Standards Review Organizations (PSROs). Although HCFA, which makes reimbursement policy, has its own research arm, the Office of Research and Demonstrations, it has seldom conducted technology assessments. NCHCT, an agency legislatively mandated to support comprehensive assessments of health care technologies for all effects (including health, economic, and social), was not funded for 1982.

Social assessment activities have been conducted by several Government mechanisms. OTA was established in 1972 as an analytic support agency to conduct policy research on science and technology issues for congressional committees. OTA's health-related reports have focused primarily on methods available for assessing technologies and issues prompted by their use. The National Commission for the Protection of Human Subjects of Biomedical and Behavioral Research was established in 1974 to develop ethical guidelines for conducting research in human subjects. The National Commission produced numerous reports with recommendations, many of

which were adopted by DHHS,* particularly those governing the protection of human subjects. The Ethics Advisory Board, which was established in 1978 at the National Commission's recommendation but was not funded in 1980, was mandated to review ethically problematic research protocols and research involving human projects. The board fielded queries from other DHHS agencies such as NIH and the Centers for Disease Control. The President's Commission for the Study of Ethical Problems in Medicine and Biomedical and Behavioral Research succeeded the National Commission in 1978. Members of the President's Commission are appointed representatives from DHHS, the Department of Defense, the Veterans Administration, the Central Intelligence Agency, the National Science Foundation, and the White House Office of Science and Technology Policy. The President's Commission conducts studies in medical practice and biomedical research and examines five subjects for legal and ethical implications: informed consent, privacy, uniform definition of death, genetic issues and unborn humans, and availability of health services. NCHCT's responsibilities, as mentioned above, included assessment of the ethical, legal, and social implications of medical technologies.

SYNTHESIS: USING INFORMATION AS THE BASIS FOR DECISIONS

Synthesis of the information generated during the testing stage of the assessment process is the necessary step to providing a convincing and responsible basis for decisions made during all phases of a technology's lifecycle.

The synthesis activities that pertain to medical technology assessment fall into two broad areas: 1) synthesis of the results of individual research studies; and 2) synthesis of a body of research findings with various concerns such as risk, social, ethical, or cost factors. The first type of synthesis addresses questions of safety, efficacy, or effectiveness of a given technology; the latter is more policy oriented, often seeking to set guidelines or standards for medical practice or reimbursement policy. The value of the latter depends, in large part, on the adequacy of the former.

Synthesis of Research Findings

The traditional approach to synthesizing research information is the literature review, an article summarizing the data of those studies a reviewer believes to be the most relevant to the topic under review. Literature reviews are useful and heavily relied on, but because of their scope and the delays in the journal publication system, such reviews are rarely timely, especially in reporting an ineffective or unsafe technology. Furthermore, the reviews are subjective and often have no commentary on methodological problems in individual studies.

More systematic procedures for integrating and interpreting sets of research evidence do exist and can be employed. The most simple technique

*Then the Department of Health, Education, and Welfare.

is a simple classification technique, sometimes called the "voting method." This technique involves selecting a sample of evaluative studies, coding some aspect of the design, classifying outcomes as favorable, neutral, or unfavorable and constructing tables of research findings. The method identifies methodological strengths and weaknesses among studies and can help determine patient populations and under what conditions they are most likely to benefit from a technology.

Meta-analysis is a technique that assesses the magnitude of treatment impact by quantitative comparison of actual study results. This method is particularly useful in assessing treatments for which a large number of studies are available and findings across studies seem to have great variability. However, it may have drawbacks with respect to sample selection.

Currently, no single technique is fully adequate for synthesizing research; however, the application of formal quantitative procedures is beginning to give a better understanding of methodological problems in research itself. Formal procedures can segregate differential outcomes according to treatment characteristics and methodological approaches. Contradictions can then be identified, analyzed, or further researched. In the performance of formal quantitative analyses, an important suggestion is that the significance of the results should be interpreted and reported in language that is useful to decisionmakers.

Synthesis of Health, Economic, and Social Effects

How, then, does one bring together and synthesize all information available about all three categories of the effects of medical technologies—health, economic, and social? Once specific information has been synthesized through various methods in each of these realms, how can a decisionmaker balance the values and interpret them into programmatic actions?

OTA's report on CEA concluded that performing an analysis of costs and benefits can be very helpful to decisionmakers, because the process of analysis gives structure to a problem, allows an open consideration of all relevant effects of a decision, and forces the explicit treatment of key assumptions. Formal techniques such as CEA can be used to aid in the synthesis of information concerning the health and economic effects of a technology. OTA found, however, that although CEA can be useful as a decision-assisting tool, it exhibits too many methodological and other shortcomings for the numerical results to be used as the basis of policy or program decisions. For example, although CEA can be used to synthesize information concerning health and economic effects, it cannot in itself adequately address social and ethical issues. These have to be addressed more fully by other means.

The most appropriate approach to any assessment is to perform it in an open forum so that assumptions and underlying values can be challenged; to identify, measure, and, to the extent possible, value all relevant benefits/effects and costs; and to present the results of the analysis as an "array" of benefits/effects and costs rather than forcing the results into a single

aggregate measure. By arraying effects in a systematic fashion, one can place the appropriate relative emphasis on given effects whether they are quantifiable or not. This technique is designed to make more explicit the health, economic, and social consequences of any decision.

Synthesis of Opinion

Synthesis of information may occasionally present a clear-cut indication of the next stage of assessment or phase of technology development. More likely, uncertainty will still predominate for decisionmakers. The uncertainty may reflect the presence of random events or may reflect a basic lack of knowledge. The former can be analyzed by various statistical techniques: decision analysis, confidence limits, computer simulation, sensitivity analysis. However, these techniques cannot actually resolve policy controversies or substitute for informed judgment.

Policy judgments may require a synthesis of opinion which can be solicited from groups and expert input. The most common format of soliciting group opinions is the unstructured conference which may involve presentations, discussions, and debates. Another informal technique is the advisory panel approach used by many Government agencies. The four best known formal techniques used in medical contexts for resolving conflicts and uncertainty are: 1) the Delphi technique, 2) the nominal group process technique, 3) the consensus development conference (NIH), and 4) a computerized knowledge base which maintains expert opinion on the state of the art of a specific topic (e.g., the Hepatitis Knowledge Base of the National Library of Medicine, NLM). Although these formal techniques produce more reliable opinion information than an unstructured conference does, evidence of effectiveness is contradictory for the Delphi and nominal group processes and sparse for the NIH and NLM processes.

DISSEMINATION OF INFORMATION

What potentially are the direct effects of dissemination of assessment information? Who should have top priority in receiving information? How should the information be disseminated? The dissemination of assessment information directly affects the development and diffusion processes of medical technologies. The consideration of whether to disseminate information is therefore weighty. If a decision is made to disseminate information because the technology is deemed either worthy or unworthy of its next phase of development, the information must reach, at a minimum, the decisionmakers involved with the technology in any aspect of its use. That audience may range from directors of R&D in private industry, to health professionals, to the general public. Reaching the audience in a timely manner requires a systematic approach to information dissemination, especially in view of the pace and quantity of information development and the lack of mechanisms for the systematic synthesis of information. In a sense, the information available is at once too much and too little.

Government Activities

The Federal Government produces, collects, and disseminates assessment information. NCHSR, for example, disseminates the results of health services research to relevant Government agencies, the research community, and other interested parties through publications, press releases, conferences, and workshops. In 1978, the legislation authorizing NCHSR was modified to require that at least $1 million or 5 percent of its budget, whichever is less, be used for dissemination activities. In response, NCHSR established a User Liaison Program to provide substantive assistance to non-Federal health care leaders concerned with critical policy issues and operational problems in the organization, administration, regulation, and delivery of health care services at State and local levels.

Monitoring NIH's dissemination activities is the responsibility of the Office for Medical Applications of Research (OMAR), established in 1978 in the NIH Office of the Director, and assisted by the OMAR Advisory Committee. One important mechanism for dissemination is the consensus development conference. The synthesis of opinion that is achieved at a consensus conference is presented in consensus statements and supporting materials which are distributed to practicing physicians, other health professionals, the biomedical research community, and the public—through a mailing list of over 21,000 names. Also, members of the press are invited to the conferences and are encouraged to publish the results. Leading medical journals and medical societies have published the consensus materials.

In conducting medical technology assessments, information from several subject areas is often required. A common need in most assessments, however, is for information from the field of biomedicine. NLM is the major Federal library resource for biomedical literature. It is the predominant creator and disseminator of biomedical bibliographic information. NLM's coverage of the health services literature is less comprehensive than its coverage of the biomedical literature, in part because relevant health services information appears in so many diverse documents.[2] Another source of information for medical technology assessments is the National Technical Information Service (NTIS). NTIS is the central repository for scientific and technical information generated by federally funded R&D projects, including those in DHHS.

Other Mechanisms

Apart from formal Federal agency activities, mechanisms for dissemination include the public media, the mail, advertising, personal contacts, the educational process, libraries, and other types of information centers. The appropriateness of any of these mechanisms depends on whether the information is to be used in assessing or marketing a medical technology. Print media, radio, and television are primary channels to the public. In addition to carrying news about medical technologies and issues, they increas-

[2]This topic is to be explored at greater length in a separate OTA technical memorandum entitled *MEDLARS and Health Information Policy,* to be published in fall 1982.

ingly tend to have health columns and special in-depth features about health technologies. For more targeted audiences, mailings are used for solicited and unsolicited information dissemination, for example, newsletters from drug companies, advertisements from product distributors, and Federal literature. Advertising of drugs occurs in all media for the public and for health professionals. A recently developed form of advertising, the video cassette, is supplied to medical facilities. Personal contacts are an especially credible source of information exchange among health professionals. These often occur formally and informally at professional meetings.

MAJOR CONCLUSIONS OF THE STUDY

In this study of medical technology assessment, OTA has reviewed the evidence and concludes overall that there is no coherent system of assessing medical technologies. There is, however, an urgent need for such a system. The following are capsule statements of OTA's conclusions about the adequacy of the present system with respect to the four stages of technology assessment presented in figure 1: identification, testing, synthesis, and dissemination.

Identification

Emerging Technologies

OTA concludes that emerging drugs and devices are adequately and appropriately identified, but that emerging medical and surgical procedures could be better identified. Overall, however, the identification of emerging technologies for assessment is not a critical weakness of the present assessment system.

New Technologies

OTA concludes that new drugs and devices are adequately identified for the purposes of assessment, but that new medical and surgical procedures are not. The most pressing need is for some routine mechanism, e.g., the reimbursement system, to identify new procedures before they are widely adopted. The reimbursement system may be the prime candidate, because coverage and payment decisions are critical points in the diffusion of many technologies. The priority-setting systems of the institutes of NIH and of other Federal research agencies (e.g., NCHSR) are adequate and appropriate for their respective mandates, but there is not an adequate similar system to fulfill the needs of operating agencies (e.g., HCFA, planning agencies). Finally, sufficient mechanisms of opportunity for identifying new technologies could be developed. Medical specialty societies could be helpful in this area.

Existing Technologies

OTA concludes that the system for identifying existing technologies in need of assessment is inadequate. The most promising possibility for identifying such technologies may be FDA's postmarketing surveillance of marketed products. In the case of existing as well as new technologies, the

priority-setting procedures of Federal research agencies may be adequate for those agencies' respective needs; however, these procedures are not adequate for the needs of operating agencies such as HCFA. And the operating agencies themselves do not adequately identify existing technologies for assessment. Medical specialty societies could be helpful in this area. Finally, NCHCT's activities of identifying nationally important priority technologies for assessment were valuable but are not currently funded. Thus, no organization is currently performing this important task.

New Applications of Existing Technologies

OTA concludes that new applications of existing technologies in need of assessment are not adequately identified. The most promising approach would seem to be the use of the reimbursement system to link the diagnosis with the use of technology. Medical specialty societies could be helpful in this area.

Testing

OTA concludes that, in general, drugs and devices are adequately tested for safety and efficacy prior to being marketed. Medical and surgical procedures, which often include the use of drugs and devices within the practice of medicine, are not well tested for either safety or effectiveness. No class of technologies is adequately evaluated for either cost effectiveness or social and ethical implications. Finally, there is no organization whose mission it is to ensure that medical and surgical procedures are assessed for safety and efficacy or to evaluate medical technologies for cost effectiveness and for social/ethical effects.

Synthesis

OTA concludes that the synthesis phase of the present system of technology assessment is unnecessarily weak, within both the private and public sectors. Research evidence regarding the safety, efficacy, and effectiveness from the use of medical technologies is seldom examined systematically and objectively. Federal agencies and private insurers and organizations set policies, guidelines, regulations, and/or make reimbursement coverage determinations, many of which profoundly affect the adoption and level of use of medical technologies. Yet, their decisions are usually based on informal, subjective, group-generated norms which tend to support the status quo. Formal, more objective techniques do exist, however, not only for evaluating research evidence but also for making decisions and setting policy. These techniques could be used more often to aid in better decisionmaking.

Dissemination

OTA concludes that better methods need to be found to communicate information about medical technologies to health practitioners, health researchers, and health policymakers.

OTA also concludes that Government-generated research reports, many of which may be important to technology assessment, are not as accessible as they could be. Finally, NLM's mission and capabilities should be examined to determine whether more Government reports and nonserial literature should be included in its data base, and whether NLM should index articles differently for researchers interested in technology assessments.

POLICY OPTIONS

The most important policy need is to bring forth a rational, systematic approach from the present multiplicity of agencies and activities to promote and coordinate medical technology assessment. This could be accomplished in any of several ways. The options listed below are divided into two broad categories: legislative and oversight. OTA finds that there are relatively few realistic legislative options necessary for Congress to consider, primarily because there is already substantial power invested in the Secretary of Health and Human Services to develop a coherent system of medical technology assessment. Thus, in most of the deficient areas noted within this report, congressional oversight may be sufficient.

Legislative Options

1. Sponsor or grant a charter to a private/public organization to undertake medical technology assessment activities.
2. Maintain the authority of, and appropriate funds for, NCHCT.
3. Change the statutes so that HCFA can selectively reimburse for experimental technologies in return for clinical data.
4. Increase funding to train researchers in methodological and statistical principles.
5. Increase efforts to train health professionals in methodological and statistical principles.

Oversight Options

6. Encourage the private sector to take the lead in assessing medical technologies.
7. Examine how Federal research institutes (e.g., NIH), agencies (e.g., NCHSR), and research programs of operating agencies within DHHS could identify technologies better when setting research agendas; and how the PSRO program and the reimbursement system could be used to more advantage for identifying technologies for assessment.
8. Continue to conduct oversight hearings concerning the duplication and fragmentation of health-related data collection activities.
9. Examine the ability of operating agencies within DHHS (e.g., HCFA) to generate sufficient information for their own decisions related to medical technologies, and examine the extent to which the Secretary of

Health and Human Services utilizes the department's other research arms (e.g., NCHSR, NIH) to procure that information in a timely manner.

10. Examine the activities, plans, and potential for elements of DHHS (e.g., NIH) in utilizing various research methods to determine the appropriate use of medical technologies.
11. Explore how research evidence could be better evaluated by Federal health agencies when recommending, setting, or implementing health policy.
12. Examine the disposition of federally generated reports to determine how accessible and useful they have been both to private and public researchers and policymakers.
13. Examine whether NLM should include more Government research reports and other nonserial literature in its MEDLARS data bases.
14. Encourage use of the powers vested in the Secretary of Health and Human Services to develop a coherent system of medical technology assessment.

NOTE: Copies of the full report "Strategies for Medical Technology Assessment," can be purchased from the Superintendent of Documents, U.S. Government Printing Office, Washington, D.C. 20402, GPO stock No. 052-003-00887-4.

General Information

Information on the operation of OTA, the nature and status of ongoing assessments, or a list of available publications may be obtained by writing or calling:

Office of Congressional and Public Communications
Office of Technology Assessment
U.S. Congress
Washington, D.C. 20510
(202) 226-2115

Publications Available

OTA Annual Report.—Details OTA's activities and summarizes reports published during the preceding year.

List of Publications.—Catalogs by subject area all of OTA's published reports with instructions on how to order them.

Press Releases.—Announces publication of reports, staff appointments, and other newsworthy activities.

OTA Brochure.—"What OTA Is, What OTA Does, How OTA Works."

Assessment Activities.—Contains brief descriptions of assessments presently under way and recently published reports.

Contacts Within OTA

(OTA offices are located at 600 Pennsylvania Avenue, S.E., Washington, D.C.)

Office of the Director	224-3695
Office of Congressional and Public Communications	224-9241
Energy, Materials, and International Security Division	226-2253
Health and Life Sciences Division	226-2260
Science, Information, and Natural Resources Division	226-2253
Administration Office	224-8712
Personnel Office	224-8713
Publications	224-8996

U.S. GOVERNMENT PRINTING OFFICE : 1982 O - 98-143

CONGRESS OF THE UNITED STATES
OFFICE OF TECHNOLOGY ASSESSMENT
WASHINGTON, D.C. 20510

OFFICIAL BUSINESS
PENALTY FOR PRIVATE USE, $300

POSTAGE AND FEES PAID
OFFICE OF TECHNOLOGY ASSESSMENT

OTA-H-182

SEPTEMBER 1982